

EXHIBIT A

05/22/00
USPTO
PENDING CLAIMS IN APPLICATION SERIAL NO: 08/475,470
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1. A recombinant adeno-associated virus vector, which comprises:

- a) at least a portion of the adeno-associated virus genome; and
- b) at least one eukaryotic based nucleic acid sequence that encodes a wild-type gene product controlled by a eukaryotic based *cis*-acting regulatory sequence chosen from the region located from about hypersensitive site I to about hypersensitive site VI of the human globin gene cluster, which is heterologous to the wild-type gene product, said virus vector having the property of regulating immune cell specific expression of said nucleic acid sequence or nucleic acid sequences upon stable transduction of a target mammalian immune cell.

8. A recombinant adeno-associated virus vector of Claim 1 wherein said eukaryotic *cis*-acting regulatory sequence is chosen from the region located within the group of *cis*-acting regulatory sequences consisting of hypersensitive site I, hypersensitive site II, hypersensitive site III, hypersensitive site IV, and hypersensitive site VI, in association with the human globin gene.

16. A recombinant adeno-associated virus vector of Claim 1 wherein said *cis*-acting regulatory sequence comprises hypersensitive site II, associated with the human globin gene cluster.

19. A recombinant adeno-associated virus vector of Claim 1 wherein said immune cell is chosen from the group consisting of a human hemopoietic stem cell, a human myeloid progenitor cell and a human erythroid progenitor cell.

20. A recombinant adeno-associated virus vector of Claim 1 wherein said immune cell is K562.

33. A recombinant adeno-associated virus vector of Claim 1 which comprises a nucleic acid sequence encoding a wild-type Fanconi anemia C complementing protein.

39. A recombinant adeno-associated virus vector of Claim 1 which comprises a nucleic acid sequence encoding a wild-type Factor IX protein.